FOR UK MEDIA ONLY

Pfizer enrols first UK participant in Phase 3 investigational gene therapy study for Duchenne Muscular Dystrophy

Walton Oaks, Pfizer UK, 11 March 2021 – Pfizer Ltd, today announced that the first UK participant has been enrolled in the Phase 3 CIFFREO study, which will evaluate the efficacy and safety of the investigational gene therapy candidate PF-06939926 in boys with Duchenne muscular dystrophy (DMD).

The first patient was enroled in The Newcastle upon Tyne Hospitals NHS Foundation Trust, one of three UK sites for the clinical trial and part of 55 globally, across 15 countries. The CIFFREO trial is expected to enrol 99 ambulatory male patients, ages 4 to 7.

Dr Michela Guglieri, Consultant Neurologist at Newcastle Hospitals NHS Foundation Trust and UK Chief Investigator, commented: "The enrolment of the first UK patient in this Phase 3 gene therapy programme is a great achievement for the UK Duchenne community and we are very pleased to contribute to innovative research in the pursuit of future therapies. There are currently no approved disease-modifying treatment options available for all genetic forms of the disease in the UK. I'm very proud to be leading the UK arm of this global study."

CIFFREO is a Phase 3 global, multicentre, randomised, double-blind, placebo-controlled study. The primary endpoint of the study is the change from baseline in the North Star Ambulatory Assessment (NSAA) at one year. The NSAA is a 17-item test that measures gross motor function in boys with DMD. Regardless of cohort, eligible participants are scheduled to receive the investigational gene therapy, either at the start of the study or after one year following treatment with placebo. Participants will be followed in the CIFFREO study for five years after treatment with the investigational gene therapy.

Jack Brownrigg, Rare Disease Medical Director, Pfizer UK said: "This is the first Phase 3 DMD gene therapy programme to begin enrolling eligible participants in the UK. We believe our gene therapy candidate, if successful in Phase 3 and approved, has the potential to significantly improve the trajectory of DMD disease progression.

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As well as a positive milestone for the DMD community, this is also an important step for the UK life science sector. This exciting development comes shortly after the UK Government launched a new Rare Disease framework which places an emphasis on improving access to speciality care, treatment and medicines. With the era of gene therapy increasingly becoming a reality, it is now more important than ever that we work together to do all we can to ensure rare disease patients have access to these treatments once deemed well tolerated and efficacious."

DMD is an X-linked disease that is caused by mutations in the gene encoding dystrophin, which is needed for muscle membrane stability. Due to the lack of dystrophin, boys present with muscle degeneration that progressively worsens with age to the extent that they require wheelchair assistance when they are in their early teens, and unfortunately, usually succumb to their disease by the time they are in their late twenties. It is estimated that there are ~140,000 boys affected with DMD worldwide.

PF-06939926 received Fast Track designation from the U.S. Food and Drug Administration in October 2020, as well as Orphan Drug and Rare Paediatric Disease designations in the United States in May 2017.

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Notes to Editors

About CIFFREO: A clinical trial for boys with Duchenne Muscular Dystrophy

CIFFREO is a Phase 3 global, multi-centre, randomized, double-blind, placebo-controlled study to assess the safety and efficacy of PF-06939926 investigational gene therapy in 99 ambulatory male participants, ages 4 through 7 years, with a genetic diagnosis of DMD who are on a stable daily regimen of glucocorticoids. The participants are negative for neutralising antibodies against AAV9, as measured by the test done for the study as part of screening.

Eligible participants will be randomized into Cohort 1 or Cohort 2. Treatment will consist of two single intravenous infusions, one of PF-06939926 and one of placebo; approximately two thirds will be in Cohort 1 and receive PF-06939926 gene therapy at the start of the study and placebo after one year, and approximately one third will be in Cohort 2 and receive placebo at the start of the study and receive gene therapy after one year, if they remain eligible. All participants will be followed in an open-label extension study for 5 years after treatment with the gene therapy. The primary endpoint of the study is a change from baseline at one year in the North Star Ambulatory Assessment (NSAA) total score. For more information, visit: clinicaltrials.gov.

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